Initial Approval: January 11, 2017
Revised Dates: April 12, 2017

CRITERIA FOR PRIOR AUTHORIZATION

Exondys 51® (eteplirsen)

PROVIDER GROUP Professional

MANUAL GUIDELINES The following drug requires prior authorization:
Eteplirsen (Exondys 51®)

CRITERIA FOR INITIAL APPROVAL (must meet all of the following):

- Diagnosis of Duchenne muscular dystrophy (DMD)
- Submission of medical records (e.g., chart notes, laboratory values) confirming the mutation of the DMD gene is amenable to exon 51 skipping
- Patient is 7 years of age or older
- Medication is prescribed by or in consultation with a pediatric neurologist
- Patient is not taking Exondys 51 with any other RNA antisense agent (e.g., drisapersen), or any other gene therapy
- Exondys 51 dosing for DMD is in accordance with the United States Food and Drug Administration approved labeling: maximum dosing of 30 mg/kg once weekly
- Patient must have a trial of deflazacort, prednisone, or prednisolone for at least 52 weeks with failure to maintain ambulation

LENGTH OF APPROVAL: 6 months

CRITERIA FOR RENEWAL:

- Documentation supports positive response to therapy (must meet all of the following):
  - Increase in dystrophin level
  - Improved 6-minute walking test
  - Improvement in respiratory or muscle strength

LENGTH OF APPROVAL: 3 months

Notes:

- The recommended dose of EXONDYS 51 is 30 milligrams per kilogram administered once weekly as a 35 to 60 minute intravenous infusion.
- Exondys 51 is unproven and not medically necessary for:
  - Duchenne muscular dystrophy with mutations that are *not* amenable to exon 51 skipping
  - Becker muscular dystrophy
  - Congenital muscular dystrophy
  - Distal muscular dystrophy
  - Emery-Dreifuss muscular dystrophy
  - Facioscapulohumeral muscular dystrophy
  - Limb-Girdle muscular dystrophy
  - Myotonic muscular dystrophy
  - Oculopharyngeal muscular dystrophy

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DRUG UTILIZATION REVIEW COMMITTEE CHAIR

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DATE

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PHARMACY PROGRAM MANAGER
DIVISION OF HEALTH CARE FINANCE
KANSAS DEPARTMENT OF HEALTH AND ENVIRONMENT

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DATE